Exhibit 10.1

CONFIDENTIAL TREATMENT REQUESTED

EXECUTION VERSION

LICENSE AGREEMENT

By and between

Généthon

and

AveXis, Inc.

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This license agreement ("**Agreement**") is effective as of March 9, 2018 ("**Effective Date**") made by and between **AveXis, Inc.**, a corporation organized under the laws of the State of Delaware, having its principal place of business at 2275 Half Day Road, Suite 200, Bannockburn, IL 60015 ("**AveXis**" or "**Licensee**") and **Genethon**, a French non-profit organization organized under the French law of July 1, 1901, having its principal place of business at 1 bis rue de l'Internationale, 91002 Evry Cedex, France ("**Genethon**"), acting in its own name and for its own behalf as well as in the name and on behalf of **Centre National de la Recherche Scientifique** (Scientific and Technological Public Institute), having its principal place of business at 3 rue Michel-Ange, 75794 Paris Cedex 16 France, and for business identification (SIRET) number: 180089013 04033 ("**CNRS**").

Each of Genethon and CNRS may be referred to in this Agreement as "**Licensor**". All rights and obligations set forth in this Agreement shall be performed by Genethon, acting in its own name and for its own behalf as well as in the name and on behalf of CNRS.

Each of Licensor and Licensee may be referred to in this Agreement individually as a "Party" or together as the "Parties."

BACKGROUND

WHEREAS, Genethon is a leading center for the development of gene therapies for rare diseases.

WHEREAS, AveXis is a clinical-stage gene therapy company focused on bringing gene therapy out of the lab and into the clinical setting for patients and families suffering from rare and orphan neurological genetic diseases.

WHEREAS, AveXis is currently developing a treatment for spinal muscular atrophy (SMA).

WHEREAS, Genethon is co-owner of the Licensed Patents as attached in Exhibit A that are necessary for the administration of such treatment.

WHEREAS, Licensor desires to grant rights under the Licensed Patents to Licensee through a license to enable the commercial Development of gene therapies in the Field.

WHEREAS, Licensee desires to obtain a license under the Licensed Patents, in order to Develop gene therapies in the Field and, if such efforts are successful, to Develop and Commercialize resulting gene therapy products.

NOW, THEREFORE, the Parties hereby agree as follows:

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ARTICLE 1. DEFINITIONS AND INTERPRETATION

1.1. Definitions

Unless the context otherwise requires, the terms in this Agreement, when used with initial capital letters, shall have the meanings set forth below or at their first use in this Agreement:

"AAV Vector" means the recombinant adeno-associated virus vector as defined in the Licensed Patents.

"**AAV9**" means (a) the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank (protein id AAS99264.1) and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector covered by a Licensed Patent.

"Abandon" (or "Abandonment") means, with respect to a Licensed Product and a country in the Territory:

(a a failure from Licensee and/or its Affiliates and/or sublicensees to initiate or conduct material Development activities or

) Commercial activities with respect to particular Licensed Product in any country of the Territory during any consecutive twelve (12) month period; provided, that such twelve (12) month period shall automatically be extended if any failure to start or conduct material Development activities is a result of any Force Majeure event or any clinical or regulatory hold imposed by the applicable Regulatory Authority or is otherwise outside the reasonable control of Licensee and/or its Affiliates and/or sublicensees; or

(b an affirmative decision by Licensee and/or its Affiliates and/or sublicensees (which shall be provided in writing to Licensor) to

) permanently discontinue all Commercialization activities with respect to a particular Licensed Product in such country; provided, that such decision shall not constitute notice of Abandonment if any decision to discontinue Commercialization activities is a result of any Force Majeure event or any clinical or regulatory hold imposed by the applicable Regulatory Authority or is otherwise outside the reasonable control of Licensee and/or its Affiliates and/or sublicensees.

"Abandonment Cure Period" means the hundred twenty (120) days period after the date of the written notice of Abandon, as provided under paragraph (a) above, from Licensor to Licensee.

"Affiliate" means, with respect to a Party, any person, corporation, firm, joint venture or other entity which directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such Party. As used in this definition, "control" means the possession of the majority of the ownership, or the power to direct or cause the direction of the management of policies, of an entity, whether through the ownership of the outstanding voting securities thereof, by contract or otherwise.

"**AFM-Téléthon**" means the French Association against Myopathies (Association Française contre les Myopathies) which is Licensor's founder and principal funder.

"Background IP" means, with respect to one Party, all Patents, Know-How and other intellectual property rights (a) owned or possessed though a license agreement by such Party as of Effective Date or (b) owned or possessed through a license agreement by such Party on or after Effective Date. Background IP does not include Licensed Patents.

"BLA" means a Biologics License Application, as defined in the U.S Public Health Service Act and the regulations promulgated thereunder.

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"Clinical Trial" means any study of a product in human subjects.

"**Commercialization**" means activities directed to marketing, promoting, distributing or selling a Licensed Product, including all activities directed to obtaining pricing approval in the Territory; and excluding Development, Manufacturing and supply of such product. "**Commercialize**" and "**Commercialized**" shall have correlative meanings.

"Confidential Information" means any confidential or proprietary information disclosed or made available in any form whatsoever by one Party (the "Disclosing Party") to the other Party (the "Receiving Party"), including this Agreement, the content of the transactions contemplated herein, all technology belonging to the Disclosing Party and any improvements thereto, any information relating to a Party's interests, business, finances, products, operations, sales, marketing, customers, suppliers and suppliers' bills of materials, trade secrets, know-how, data, processes, methods, protocols, techniques, specifications, formulas, test data, calibration information, presentations, analyses, studies, regulatory communications, patent applications (as long as unpublished and/or undisclosed), financial data, product development, assays, strategic and market research information, other relevant marketing information, clinical data and any other information, whether developed in connection with this Agreement or not and whether marked as confidential or not.

"**Development**" means, with respect to a product, research and any and all processes and activities conducted to obtain and maintain Marketing Authorization for a product, including pre- and post-marketing approval clinical studies and activities relating to development or preparation of such product for Commercialization. Development includes performance of Clinical Trials. "**Develop**" shall have the correlative meaning.

"Effective Date" shall have the meaning in the third page.

"Expert" has the definition set forth in Section 5.3.3.

"**Field**" means the treatment based on *in vivo* gene replacement therapy using an AAV9 Vector to deliver the SMN gene addressing and restricted to Spinal Muscular Atrophy ("**SMA**") in humans.

"First Commercial Sale" means, with respect to a Licensed Product and a country, the first sale for monetary value for use or consumption by the end user, within a country of the Territory, of such Licensed Product while covered by at least one Valid Claim of a Licensed Patent in such country after regulatory approval for commercialization (including pricing and reimbursement if applicable) has been obtained for such Licensed Product in such country. Treatment IND sales and compassionate use sales shall not be construed as First Commercial Sale. First Commercial Sale may occur directly from Licensee or through any of its Affiliates or sublicensees.

"Force Majeure" has the definition set forth in Section 17.5.

"Invention" means any new and useful process, article of manufacture, compound, composition of matter, formulation, or apparatus, or any improvement thereof, discovery or finding, whether or not patentable created, generated or Developed by or on behalf of one Party using the Licensed Patents, during the Term. Invention does not include Background IP and Licensed Patents.

"Know-How" means any tangible and intangible information, data, results (including pharmacological, research and development data and reports), and trade secrets, rights in data, materials, discoveries,

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improvements, compositions of matter, cell lines, assays, sequences, processes, methods, knowledge, protocols, formulas, utility, formulations, Inventions, and all other scientific, pre-clinical information or data.

"**Licensed Patents**" mean the Patents that are listed in Exhibit A. For the avoidance of doubt, the Licensed Patents' status as stated in Exhibit A will include any rights listed in the definition of Patents derived from the Licensed Patents.

"**Licensed Product(s)**" means any human therapeutic product in the Field using or having used at least one Valid Claim of the Licensed Patents whether this product is Developed, Manufactured or Commercialized by AveXis, AveXis' Affiliates and/or AveXis' sublicensees.

"**Licensee Publication**" means any public announcement, public presentation, medical communication related to the Licensed Product, where Licensee believes it is appropriate and reasonable to do so and in all cases where Licensee includes the names of its other licensing partners.

"Losses" means any liability or expense (including reasonable legal expenses, costs of litigation and attorneys' fees), damages, or judgments, whether for money or equitable relief.

"Manufacture" means activities directed to the manufacture, receipt, incoming inspections, storage and handling of raw materials and the manufacture, processing, formulation, packaging, labeling, warehousing, quality control testing (including in-process release and stability testing), supplying, shipping and release of a product, as the case may be and to the extent applicable, including manufacturing process development, scale-up and validation. "**Manufacturing"** and "**Manufactured**" shall have the correlative meaning.

"Marketing Authorization" means the technical, medical and scientific licenses, registrations, authorizations and approvals (including supplements and amendments, pre and post-approvals, pricing approvals and labeling approvals) of any Regulatory Authority necessary for the Commercialization of a product in the Field in such Regulatory Authority's jurisdiction in the Territory.

"Marketing Authorization Application" or **"MAA"** means an application for regulatory approval in order to market and/or sell a Licensed Product in any country other than the U.S.

"**Net Sales**": means the total gross receipts from sales or other dispositions of Licensed Products by or on behalf of Licensee or its Affiliates or any sublicensee to unaffiliated Third Parties for end use, less the following deductions that are documented as attributable to the Licensed Products: (a) costs actually incurred for transportation, including packing costs, freight out and insurance costs; (b) rebates, trade, quantity and wholesaler and cash discounts in amounts customary in the trade to the extent actually granted; (c) returns, chargebacks, credits and allowances actually granted; and (d) sales, taxes or excise duties actually paid. No deductions shall be made for commissions paid to individuals, whether they are with independent sales agencies or regularly employed by Licensee, or sublicensees and on its payroll, or for the cost of collections. Sales between or among Licensee and its Affiliates or any sublicensees shall be excluded from the computation of Net Sales, except where such Affiliates or sublicensees. In the event consideration other than cash is paid to Licensee, its Affiliates or any sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of transaction, as determined jointly by the Parties based on transactions of a similar type and standard industry practice, if any. Notwithstanding anything to the contrary herein, the computation of Net Sales shall not include sales of

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Licensed Products for early access programs, named patient programs, compassionate use programs or similar uses.

"Patent" means (a) any patent, re-examination, reissue, renewal, extension, supplementary protection certificate and term restoration, any confirmation patent or registration patent or patent of addition based on any such patent, (b) any pending application for patents, including provisional, converted provisional, continuations, continuations-in-part, divisional and substitute applications, and inventors' certificates, (c) all foreign counterparts of any of the foregoing, and (d) all applications claiming priority to any of the foregoing.

"Pivotal Clinical Trial" means: (a) a Clinical Trial that would satisfy the requirements for a Phase 3 study as defined in 21 CFR § 312.21(c) (or successor regulation); or (b) any other Clinical Trial that the applicable Regulatory Authority has agreed is sufficient to form the primary basis of an efficacy claim in an MAA submission, including any such study that is determined to have become pivotal after its commencement.

"Reasonable Efforts" means the efforts and resources normally used by a company in the biopharmaceutical industry of similar size and resources as Licensee for a product that is of similar market potential at a similar stage in its development (before Market Authorization) or product life (after Market Authorization), taking into account all relevant factors, including the potential profitability of the Licensed Products, the costs and risks of Developing, Manufacturing and Commercializing the Licensed Product, scientific, safety and regulatory concerns, product profile, the competitiveness of the marketplace and the proprietary position of the Licensed Products.

"Regulatory Authority" means, in a particular country or jurisdiction in the Territory, any applicable governmental authority involved in granting (a) approval to initiate or conduct clinical testing in humans,

(b) the authorizations, approvals, licenses, permits, consents, registrations and filings necessary for the Commercialization of a product in a country in the Territory including Marketing Authorizations and manufacturing licenses, or (c) to the extent applicable in such country or jurisdiction, pricing approval for a product in such country or jurisdiction.

"**Term**" has the definition set forth in the Section 12.1.

"Territory" means worldwide.

"Third Party" means any party other than a Party.

"Treatment IND" means an Investigational New Drug treatment used for Clinical Trial before the drug has been approved.

"Valid Claim" means, with respect to any country, a claim of (a) an issued/granted and unexpired patent (as may be extended through supplementary protection certificate or patent term extension or the like); and/or (b) a pending patent application that has not been pending for more than five (5) years from the Effective Date, in each case of (a) and (b) to the extent such claim has not been revoked, held invalid or unenforceable by a patent office, court or other governmental agency of competent jurisdiction in a final and non-appealable judgment (or judgment from which no appeal was taken within the allowable time period) and which claim has not been disclaimed, denied or admitted to be invalid or unenforceable through reissue, re-examination or disclaimer or otherwise.

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1.2. Interpretation

The captions and headings to this Agreement are for convenience only, and are to be of no force or effect in construing or interpreting any of the provisions of this Agreement. Unless specified to the contrary, references to Sections or Exhibits shall refer to the particular Sections or Exhibits of or to this Agreement and references to this Agreement include all Exhibits hereto. Unless context otherwise clearly requires, whenever used in this Agreement:

- a. the words "include" or "including" shall be construed as incorporating, also, "but not limited to" or "without limitation;"
- b. the word "day," "quarter" or "year" (and derivatives thereof, e.g., "quarterly") shall mean a calendar day, calendar quarter or calendar year unless otherwise specified (and "annual" or "annually" refer to a calendar year);
- c. the word "notice" shall mean notice in writing (whether or not specifically stated) and shall include notices, consents, approvals and other written communications contemplated under this Agreement;
- d. the word "hereof," "herein," "hereby" and derivative or similar word refers to this Agreement (including any Exhibits);
- e. the word "or" shall have its inclusive meaning identified with the phrase "and/or;"
- f. the words "will" and "shall" shall have the same obligatory meaning;
- g. provisions that require that a party or the parties hereunder "agree," "consent" or "approve" or the like shall require that such agreement, consent or approval be specific and in writing, whether by written agreement, letter, approved minutes or otherwise;
- h. words using the singular or plural number also include the plural or singular number, respectively.

ARTICLE 2. <u>GRANT OF RIGHTS</u>

2.1. Licensor hereby grants to Licensee, subject to the terms and conditions of this Agreement, an exclusive, sub-licensable, non-transferable (except in accordance with Section 17.3), royalty and milestone bearing license under the Licensed Patents to Develop, to Manufacture, to Commercialize or to import any Licensed Products for such purpose in the Territory, solely in the Field.

2.2. Nothing in this Agreement shall be interpreted as a transfer of Licensor ownership rights in the Licensed Patents to Licensee or any of Licensee's Affiliates or Licensee's sublicensees. Licensor shall remain the sole owner of the Licensed Patents.

2.3. Notwithstanding anything to the contrary in this License Agreement, Licensor may use and permit any Third Party to use the Licensed Patents for (a) noncommercial-research or teaching purposes in the Field limited to a collaboration with another academic or not-for-profit research organization or on its own; and (b) any purposes outside of the Field, particularly but not restricted to other therapeutic indications and commercial purposes.

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ARTICLE 3. <u>SUBLICENSING</u>

3.1. Licensee may enter into non-sublicensable sublicensing agreements under the Licensed Patents with a Third Party, subject to prior information of Licensor of the identity of said Third Party and of the business terms of such sublicensing agreement.

3.2. Licensee agrees that any sublicenses granted by it under the Licensed Patents shall contain terms and conditions in compliance with the terms of this Agreement, and any obligations of this Agreement applicable to sublicensees. In particular, each sublicense agreement shall have a provision providing that, in the event of termination of this Agreement for any reason, Licensor and sublicensee shall enter into good faith negotiations to conclude a direct license under the terms of this Agreement so long as such termination did not arise directly or indirectly as a result of such sublicensee.

Licensee guarantees performance and acceptance of the obligations and commitments set forth in this Agreement and in the sublicense agreement of each and any of its sub-licensees.

3.3. Licensee agrees to forward to Licensor (and to obtain agreement of sub-licensee to do so) a complete copy of each fully executed sublicense agreement postmarked within thirty (30) days of the execution of the sublicense agreement. To the extent permitted by law, Licensor agrees to maintain such sublicense agreement in confidence.

ARTICLE 4. LICENSEE UNDERTAKINGS AND PERFORMANCE

4.1. <u>Diligence</u>. Licensee shall use its Reasonable Efforts to Develop and Commercialize, in the European Union (including the UK), in particular in France, and the USA, a Licensed Product in the Field.

4.2. <u>Manufacture and Commercialization of Licensed Products</u>. Licensee shall be responsible for the Manufacture and Commercialization of the Licensed Products.

4.3. <u>Clinical Trials</u>. Licensee agrees to make in France Clinical Trials on patients suffering from Spinal Muscle Atrophy and to retain "Association Institut De Myologie" (whose founders are the AFM-Téléthon and Genethon) as an investigative site to the extent it is regulatory possible. The clinical trial agreement shall be negotiated on commercially reasonable terms, directly by and between the "Association Institut De Myologie" and Licensee, and the parties shall execute such agreement before December 31th, 2019.

4.4. <u>Approval from Regulatory Authorities</u>. Licensee shall have the responsibility to prepare and file all requested documentation to obtain and maintain approval from the Regulatory Authorities (including Marketing Authorizations) necessary for the Development and Commercialization of the Licensed Products in the Field and the Territory, and otherwise interact with the relevant authorities as appropriate with respect to the Licensed Products. This includes in particular (i) the drafting of a protocol, application for clinical trial approval, interviewing and identifying clinical centers, appointing a CRO or recruiting patients ; (ii) the analysis of data generated from any trial or manufacturing; (iii) the preparation of regulatory filings for the Licensed Product or preparing briefings for such regulatory filings; and (iv) meetings with regulatory authorities.

4.5. French Patient Access. Following the appropriate regulatory approvals, Licensee will use Reasonable Efforts to make available within France all the Licensed Products indicated for SMA at prices that would allow appropriate reimbursement scheme and that would not constitute an obstacle for patients

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to have access to the therapy. Licensee shall be solely responsible for designing and conducting all Commercialization activities necessary to fulfill its obligations under this Section 4.5.

4.6. <u>Process Development.</u> Upon Licensor's request, Licensee agrees to discuss in good faith with Licensor regarding a collaboration concerning the Development of cells in suspension process, in or outside the Field.

4.7. License with ReGenX Biosciences. Upon Licensor's request, Licensee will provide either to Licensor or to its Affiliates a worldwide, royalty-bearing, non-exclusive license under the AAV9 Patents controlled by Licensee for the SMArd indication under its current license with ReGenX Biosciences, LLC ("ReGenX"; such agreement, the "ReGenX Agreement"). This license agreement shall be subject to the terms of the existing license with ReGenX, including but not limited to the royalty obligations and milestone obligations to ReGenX and the prohibition on further sublicensing.

4.8. Licensee agree to provide Licensor with with **five hundred thousand US dollars (\$500,000)** on an annual basis, for **three (3) years**, to support the conduct of novel gene therapy research by Licensor in the field of SMA. Such payment shall be made on the signing date of this Agreement and thereafter upon each anniversary date of the Agreement. Licensor will provide annual report on the R&D work performed. In consideration of such compensation, should the conduct of such novel gene therapy research by Licensor in the field of SMA results in a Patent, Licensor shall first propose to Licensee a license on such patent, provided that Licensee is in compliance with the terms of this Agreement. Licensee shall have a time limit of thirty (30) calendar days from receiving the request from Licensor, giving details of the proposed license, to notify its acceptance or refusal of the license. If Licensee declines such license or does not respond to Licensor in due time, Licensor shall be free to propose and conclude such license with any third party of its choice, without any further notification to be given to Licensee.

ARTICLE 5. <u>FINANCIAL CONSIDERATION</u>

In consideration for the exclusive license granted to Licensee and Licensor's commitments pursuant to this Agreement, Licensee shall pay Licensor the following:

5.1. License Fees

In consideration for the license rights granted to Licensee under this Agreement, Licensee shall pay Licensor:

- (i) a one-time, non-refundable, non-creditable amount of four million US dollars (\$4,000,000 USD), which shall be paid to Licensor at signing of this Agreement.
- (ii) a nonrefundable, ongoing annual fee of twenty-five thousand US dollars (\$25,000 USD) upon each anniversary date of the Agreement to cover Licensor's management fees of this Agreement.

5.2. Milestone Payments

Licensee agrees to pay to Licensor during the Term, the one-time, non-refundable, non-creditable Milestone Payments set forth in the table below, upon first achievement, by Licensee alone or together with its Affiliates and/or a sublicensees of the applicable milestones with respect to the Licensed Product which first achieves such applicable milestone:

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* 1 _{st} Milestone: 1 _{st} treatment in EU Pivotal Clinical Trial	Two million US dollars (\$2,000,000 USD)
* 2nd Milestone: 1st US BLA or EU MAA submission	Two million US dollars (\$2,000,000 USD)
* 3 _{rd} Milestone: 1 _{st} USA BLA or EU MAA approval	Two million US dollars (\$2,000,000 USD)
* 4 th Milestone: annual world-wide net sales equal five hundred million US dollars (\$500,000,000 US	Two million US dollars (\$2,000,000 USD)
* 5 th Milestone: annual world-wide net sales equal one billion US dollars (\$1 000 000 000 USD)	Three million US dollars (\$3,000,000 USD)
Total	Eleven million US dollars (\$11,000,000 USD)

For purpose of the above the terms "net sales" shall have similar meaning as the definition of "Net Sales" but shall include the sales of Licensed Products and the sales of identical products not covered by a Valid Claim in a given country of the Territory (hereinafter referred to as "**Identical Products**").

5.3. <u>Royalties on Net Sales</u>

5.3.1. As of the First Commercial Sale, a **five percent (5%)** Royalty shall be paid on Net Sales on a country-by-country and Licensed Product-by-Licensed Product basis. For each country and each Licensed Product, such Royalties shall start as of the First Commercial Sale of the Licensed Product in the country and shall end at the later of: (a) expiration of the last Valid Claim of an issued Licensed Patent covering such Licensed Product inside the Field in in such country or (b) ten years from First Commercial Sale of such Licensed Product in the Country (the "**Royalty Term**").

Notwithstanding the foregoing:

- Royalties shall be reduced by 50%, on a Licensed Product-by-Licensed Product and country- by-country basis, should a Licensed Product not be covered anymore by a Valid Claim in the concerned country of the Territory during the Royalty Term provided above. Such reduction shall only apply to the sales of such Licensed Product concluded after the expiration of the last Valid Claim of an issued Licensed Patent covering such Licensed Product inside the Field in such country.
- In addition, Royalties shall not be payable in the event that a Licensed Product is not covered by a Valid Claim in the country of its sale at the time of First Commercial Sale of such Licensed Product in such country of the Territory, and for so long as a Valid Claim continues not to exist.
- In addition and notwithstanding the foregoing, should the Licensed Patent No15/713,347 filed on September 22, 2017 (the US continuation) referred in the Exhibit A as being still "pending" at the Effective Date, be refused by the US patent office before the first commercial sale of a product using Licensor intellectual property rights that were intended to be protected by Licensed Patent application No15/713,347, Licensee shall pay to Licensor a fee amounting to two and a half percent (2.5%) of the net sales of such products in the USA for a duration of ten years from such first commercial sale of such product in the USA. For the purpose of the above, the terms "first commercial sale" and "net sales" shall have similar meaning as the definition of these terms when applied to Licensed Products.

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5.3.2. <u>Royalty Monetization ROFR</u>. In the event Licensor seeks to monetize a portion or the entirety of its royalty obligation under this Agreement, Licensor shall inform Licensee to allow the Licensee to make an offer. Licensor shall give Licensee a thirty (30) days period to submit an offer prior to make a decision with respect to such royalty monetization.

5.3.3. The Parties will seek to negotiate any dispute between them about Net Sales in good faith. Any dispute between the Parties about Net Sales in any instance, which has not been resolved by the Parties with twenty (20) Business Days, may, at the request of either Party, be referred to an expert. The expert will be a single, independent chartered accountant to be agreed between the Parties, or in default of agreement between the Parties within five (5) Business Days, to be selected at the request of either of them by the Brussels (Belgium) Court ("**Expert**"). Any dispute to be referred to the Expert will be decided upon in a final and binding manner by the Expert acting as a technical expert and not as an arbitrator. Any actions, decisions, awards or payments to be made or taken pursuant to the determination of the Expert will be made or taken within thirty (30) Business Days of notification of the same to the relevant Parties. The costs of the Expert will be borne by the Parties as determined by the Expert.

5.4. Sublicense Fees

5.4.1. In addition to milestones that Licensee shall pay to Licensor as per Section 5.2 in case of achievement of such milestones by Licensee (together with its Affiliates and its sublicensees) and in addition to the royalties that Licensee shall pay to the Licensor as per Section 5.3, Licensee shall pay to Licensor a percentage of any non-royalty sublicense fee (including upfront payments, milestone payments and profit share payments) received by Licensee from any sublicensee following execution of a sublicense agreement for a Licensed Patents, after deducting any non-royalty fee mentioned above from milestone payments that Licensee owes Licensor pursuant to Section 5.2 for the same milestone event.

5.4.2. The applicable percentage due to Licensor for each sublicense shall be fifteen percent (15%).

5.5. <u>Royalty Stacking</u>

5.5.1. If, in connection with the Manufacture, use, or Commercialization of a Licensed Product, Licensee is obligated to pay royalties to Licensor and any Third Parties that, in the aggregate, exceed **fifteen percent (15%)** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

$$R = (C^{*}(A/(A+B)))$$

Where:

R = Reduction of Licensor royalty, A = Unreduced Licensor royalty,

B = sum (in percentage) of all Third Party royalties, and C = increment of projected total royalty above 15%.

5.5.2. Notwithstanding the foregoing, Licensee will pay to Licensor no less than **75%** of the royalties that Licensee would otherwise pay to Licensor if there were no royalties due to Third Parties.

5.6. Payment Method

Payments under this Agreement shall be paid in US Dollar by wire transfer, or electronic funds transfer (EFT) of immediately available funds to:

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Account owner:	Généthon
Bank name & address:	BNP Paribas, 37-39 rue d'Anjou, 75008 Paris, France
Bank Code:	XXXXX
Agency Code:	xxxxxx
Account No.:	xxxxxxxxx
Key:	XX
IBAN:	xxxxxxxxxxx
BIC:	xxxxxxxxxxxx

Any value, taxes, or other expenses incurred in the transfer shall be paid entirely by Licensee.

All sums due under this Agreement are exclusive of any value added tax which will be payable in addition by Licensee on the issuing by Licensor of an appropriate value added tax invoice.

5.7. Late payment

Should Licensee fail to make any royalties and other payments, including patent expense reimbursements, required to be paid by Licensee pursuant to this Agreement, on the due date, Licensor reserves the right, without any further notification being given by Licensor:

- (a) to suspend and/or cancel any of its obligations; and/or
- (b) to charge interest calculated on a day by day basis, as from the due date of payment, and equal to higher of (i) three (3) times the French legal interest and (ii) the last European Central Bank rate plus ten (10) points; and/or
- (c) to terminate the Agreement by right in accordance with Section 12.2.1.

The interest payment shall be due from the day the original payment (or portion thereof) was due until the day that such payment was received by Licensor. The payment of such interest shall not restrict Licensor from exercising any other rights it may have because any payment is overdue.

ARTICLE 6. INTELLECTUAL PROPERTY

6.1. Background IP

As between the Parties, Licensor and Licensee shall remain sole owners of their respective Background IP. For the avoidance of doubt, this Agreement does not confer any rights, titles or interests of a Party's Background IP to the other Party.

6.2. <u>Inventions</u>

As between the Parties, Licensor and Licensee shall remain sole owners of their respective Inventions. For the avoidance of doubt, this Agreement does not confer any rights, titles or interests of a Party's Invention to the other Party.

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ARTICLE 7. CONFIDENTIALITY

7.1. <u>Confidentiality; Exceptions</u>

Except to the extent expressly authorized by this Agreement or otherwise agreed by the Parties in writing, during the term of this Agreement and for ten (10) years thereafter (except that with respect to Confidential Information that constitutes a trade secret, the Recipient's obligations under this Agreement will continue with respect to such trade secret for as long as such information remains a trade secret), the Parties agree that the Receiving Party shall keep confidential and shall not publish or otherwise disclose or use for any purpose other than as provided for in this Agreement any Confidential Information furnished to it by the other Party pursuant to this Agreement, regardless of whether such information is specifically designated as confidential and regardless of whether such information is in oral, written, electronic or other form.

Notwithstanding the foregoing, Confidential Information shall not be deemed to include information or materials to the extent that it can be established by written documentation by the Receiving Party that such information or material:

(a was already known to or possessed by the Receiving Party or any of its Affiliates, other than under an obligation of

-) confidentiality (except to the extent such obligation has expired or an exception is applicable under the relevant agreement pursuant to which such obligation is established), at the time of disclosure;
- (b) was generally available to the public or otherwise part of the public domain at the time of its first disclosure to the Receiving Party or any of its Affiliates, except by breach of this Agreement;
- (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party or any of its Affiliates in breach of this Agreement;
- (d) was independently developed by the Receiving Party or any of its Affiliates as demonstrated by documented evidence prepared contemporaneously with such independent development; or
- (e) was disclosed to the Receiving Party or any of its Affiliates, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

7.2. <u>Authorized Use and Disclosure</u>

The Receiving Party shall receive, maintain, and hold the Confidential Information in strict confidence; and exercise the same degree of care as it shall exercises to safeguard its own information but in no event less than reasonable care. Each Party may use and disclose Confidential Information of the other Party as follows:

(a under appropriate confidentiality provisions substantially equivalent to those in this Agreement, in connection with the

) performance of its obligations or exercise of rights granted to such Party in this Agreement; however, the Receiving Party will be responsible for any disclosure or use of Confidential Information of the Disclosing Party made by any person to

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whom the Receiving Party disclosed such Confidential Information as though such disclosure or use was conducted by the Receiving Party; and

(b to the extent such disclosure is reasonably necessary in prosecuting and maintaining Patents (including applications therefor) in

) accordance with this Agreement, prosecuting or defending litigation, complying with applicable governmental regulations, conducting Development or Commercialization hereunder, obtaining and maintaining Marketing Authorizations, or otherwise required by law applicable to such Party or a Party's disclosure under regulations promulgated by applicable security exchanges; provided however, that if a Party is required by applicable law or under security exchange rules it will, except where prohibited by applicable law or impracticable, give reasonable advance notice to other Party of such disclosure requirement and, where practicable, will use its reasonable efforts to secure confidential treatment of such Confidential Information required to be disclosed.

Either Party may disclose to *bona fide* potential investors, lenders and acquirors/acquirees, and to such Party's consultants and advisors, the existence and terms of this Agreement to the extent necessary in connection with a proposed equity or debt financing of such Party, or a proposed acquisition or business combination, or to *bona fide* potential sublicensees, so long as such recipients are bound in writing to maintain the confidentiality of such information in accordance with the terms of this Agreement.

Notwithstanding the forgoing, Licensor may disclose any Licensee Confidential Information to the CNRS as co-owner of the Licensed Patents, subject to obligations of confidentiality and non-use at least as restrictive as those set forth in this Section 7.

ARTICLE 8. <u>RECORD KEEPING – AUDIT</u>

8.1. Licensee agrees to keep, and will ensure that each of its Affiliates and sub-licensees keeps, accurate and correct records of Licensed Products Developed, Manufactured and Commercialized under this Agreement appropriate to determine the amount of royalties due to Licensor. These records shall be retained for at least five (5) years following a given reporting period.

8.2. Records shall be made available by Licensee, its Affiliates and sub-licensees during normal business hours for inspection, by an accountant or other designated auditor selected by Licensor for the sole purpose of verifying reports, milestones achievements and royalty payments hereunder. The accountant or auditor shall only disclose to Licensor information relating to the accuracy of reports, milestones achievements and royalty payments made under this Agreement or under the agreement concluded between Licensee and its Affiliates or sub-licensees for the purpose of the Development, Manufacture or Commercialization of the Licensed Product.

8.3. Subject to Section 8.1 and 8.2, Licensor can demand at any time to access the elements of the special accounting allowing the evaluation of the commercial transactions made under the present Agreement.

8.4. Audit shall be made at the expense of Licensor, except when an inspection shows an underreporting or underpayment in excess of three percent (3%) for any twelve (12) month period. In such case, Licensee shall reimburse Licensor for the cost of the audit at the time Licensee pays the unreported milestones or the unreported royalties, including any additional payment and interest as required by Section 5.7.

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8.5. All payments required under this ARTICLE 8 shall be due within forty five (45) days of the date Licensor provides Licensee notice of the payment due.

ARTICLE 9. REPORTS ON PROGRESS, SALES AND PAYMENTS

9.1. Licensee shall provide written annual reports on its Licensed Products Development progress or efforts to Manufacture or Commercialize for the Field within sixty (60) days after December 31 of each calendar year. These progress reports shall include, but not be limited to: progress on research and Development, status of applications for regulatory approvals, Manufacture and status of sublicensing, marketing, importing, and sales during the preceding calendar year, as well as, plans for the present calendar year.

9.2. If reported progress differs from that projected between the Parties and in Milestones, Licensee shall explain the reasons for such differences. Licensee agrees to provide any additional information reasonably required by Licensor to evaluate Licensee's performance under this Agreement.

9.3. Licensee shall report to Licensor the dates for achieving milestones, the First Commercial Sale of each Licensed Product and the first commercial sale of each Identical Product in each country of the Territory within thirty (30) days of such occurrences.

9.4. Licensee shall submit to Licensor, within forty (40) days after each calendar half year ending June 30 and December 31, a royalty report setting forth for the preceding half year period the amount of the Licensed Products and Identical Products sold by or on behalf of Licensee in each country within the Territory, the Net Sales and net sales of Identical Products, and the amount of royalty accordingly due.

9.5. With each royalty report, Licensee shall submit payment of earned royalties due, in conformity with Section 5.3 and Section 5.5. If no earned royalties are due to Licensor for any reporting period, the written report shall so state. The royalty report shall be certified as correct by an authorized officer of Licensee and shall include a detailed listing of all deductions made to determine Net Sales made under ARTICLE 5 to determine royalties due.

9.6. Licensee agrees to forward semi-annually to Licensor a copy of these reports received by Licensee from its sublicensees during the preceding half year period as shall be relevant to a royalty accounting to Licensor by Licensee for activities under the sublicense.

ARTICLE 10. PROSECUTION AND MAINTENANCE

10.1. Each Party shall have the right, but not the obligation, at its sole expense, to prosecute and maintain Patents solely owned by such Party, including its own Background IP and its own Invention. Licensor shall notify Licensee in writing of material aspects of the prosecution of Licensed Patents related to the Field, and shall reasonably consider Licensee's comments with respect thereto in good faith.

10.2. Subject to Section 10.3, Licensee shall reimburse Licensor for all Third Party patent costs incurred for each Patent of the Licensed Patents as from the Effective Date and until termination of the Agreement or expiry of the last Valid Claim of the Licensed Patents. The sum reimbursed shall be creditable on an annual basis against the annual fee provided in Section 5.1(ii).

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10.3. If the Licensed Patents are licensed by Licensor to Third Parties, outside of the Field, the Third Party Patent costs incurred for the Licensed Patents as from the effective date of such additional licenses will be divided by the number of licensees, including Licensee.

10.4. Patent costs shall include all Third Party costs relating to the Licensed Patents drafting, filing, prosecution, and maintenance in all countries, including ex parte re-examination, grant, re-issuance and validation in all countries.

ARTICLE 11. INFRINGEMENT AND ENFORCEMENT

11.1. Validity challenge against Licensed Patent

11.1.1. In the event a Third Party initiate an action challenging the validity of any of the Licensed Patents ("Third Party Challenge"), Licensor agrees to notify Licensee of such an action. Third Party Challenge shall include but not be limited to declaratory judgment action, opposition, post-grant proceedings, interferences, inter-partes review, inter-partes re-examination and patent validity challenges against national or regional courts.

11.1.2. Unless the Parties otherwise agree in writing, Licensor shall have the right, but not the obligation, to defend any Third Party challenge related to the Licensed Patents, and Licensee shall reasonably assist Licensor and cooperate in any such litigation at Licensor's request and at Licensee's expenses. Licensor shall keep Licensee reasonably informed with respect to the progress of any such litigation.

11.1.3. If Licensor does not exercise its right to defend any Third Party challenge related to the Licensed Patents within the Field within a commercially reasonable period of time, then Licensee shall have such right to defend such Third Party challenge that would limit Licensee's rights in the Field, and Licensee agrees to keep Licensor reasonably informed of all material developments in connection with such Third Party challenge. Licensee shall not settle or otherwise make any admissions in connection with such Third Party challenge affect Licensor's rights or interests, without Licensor's prior written consent.

11.1.4. Licensee shall reimburse Licensor for all Third Party costs incurred due to Third Party Challenges related to the Field.

11.2. Infringement of Licensed Patents by Third Parties

11.2.1. Licensor and Licensee agree to notify each other promptly of each infringement or possible infringement of the Licensed Patents, as well as of any facts which may affect the validity, scope, or enforceability of the Licensed Patents of which either Party becomes aware.

11.2.2. Licensor may enforce any Licensed Patents with respect to Third Party infringements, at its sole discretion and at its sole expense. Licensor shall inform Licensee of its decision regarding such enforcement.

• In case of such enforcement action brought by Licensor, Licensee shall be entitled to join such action at its own costs and expenses. Licensor shall remain free to withdraw its action at any time during the procedure.

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• In case Licensor does not bring such enforcement action, Licensee may enforce any Licensed Patents with respect to Third Party infringements at its own costs and expenses only after having obtained Licensor's written approval which shall not be unreasonably withheld with regards to Licensor own interests. Licensor shall be entitled to join such action at its own costs and expenses.

11.2.3. Licensee shall reimburse Licensor for all costs incurred due to such action if the infringement related to the Licensed Patents by Third Parties relates to the Field.

11.2.4. Any damages or other compensation, monetary or not, shall be collected by Licensor. In case Licensee joins any action to enforce Licensed Patents initiated by Licensor, it may request and collect damages or other compensation for its own prejudice.

ARTICLE 12. TERM, TERMINATION AND MODIFICATION OF RIGHTS

12.1. <u>Term</u>

This Agreement commences on the Effective Date and shall continue on a Licensed Product-by-Licensed Product and country-bycountry basis until the expiration of the Royalty Term with respect to such Licensed Product in such country (the "**Term**").

12.2. <u>Termination</u>

12.2.1. Termination for cause

12.2.1.1 Either Party may terminate this Agreement entirely, effective upon written notice to the other Party, if the other Party materially breaches this Agreement and fails to cure such breach within ninety(90) days after receiving written notice thereof.

12.2.1.2 While the following list cannot be construed to limit Licensor's right to terminate the Agreement entirely in case of a material breach by Licensee of the Agreement under Sections 12.2, Licensor shall in particular have the right to terminate this Agreement on a country-by-country basis and Licensed Product- by-Licensed Product basis or in its entirety, effective upon written notice to Licensee, if Licensee fails to cure such breach within ninety (90) days after receiving written notice thereof:

- (a) if Licensor determines that Licensee has willfully made a false statement of, or omitted, a material fact in any report required by this Agreement, including under Article 9;
- (b) if Licensor determines that Licensee is not keeping Licensed Products reasonably available to the public after commercial use commences in application of Section 4.5;
- (c) if Licensor determines that Licensee cannot reasonably justify a failure to comply with undertakings provided for in Section 4.5;
- (d) if Licensee does not enter into the clinical trial agreement mentioned in Section 4.3;
- (e) if the Clinical Trial contemplated in such clinical trial agreement mentioned in Section 4.3 is not entirely performed for reason attributable to Licensee;

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(f) if Licensee fails to make any royalties and other payments, including patent expense reimbursements, required to be paid by Licensee on the due date pursuant to ARTICLE 5.

12.2.1.3 Notwithstanding the foregoing, in the event that a breach by Licensee pursuant to Section 12.2.1.1 or 12.2.1.2 would reasonably require more than ninety (90) days to cure, and Licensee provides Licensor with a reasonable plan for diligently curing such breach, such ninety (90)-day cure period shall be extended for the amount of time set forth in the plan not to exceed an additional ninety (90) days.

12.2.2. Abandonment

12.2.2.1 On a Licensed Product-by-Licensed Product and country-by-country basis, Licensor may terminate the license under Section 2.1 with respect to a Licensed Product in a given country in the event of Abandonment by Licensee with respect to such Licensed Product in such country by giving written notice to Licensee. If such Abandonment has been cured by Licensee within the Abandonment Cure Period, such termination shall not occur. If such Abandonment has not been cured by Licensee within the Abandonment Cure Period then Licensor shall be entitled at its own discretion to (i) terminate the license under Section 2.1 entirely with respect to such Licensed Product in such country, with immediate effect upon delivery to Licensee of a written notice, or to (ii) modify the license grant in removing the exclusivity granted by Licensor to Licensee under the Licensed Patents in the Field with respect to such Licensed Product in such country, with immediate effect upon delivery to Licensee of a written notice.

12.2.2.2 Notwithstanding anything in this Section 12.2, in the event Licensee fails to achieve a First Commercial Sale in EU or in the USA before December 31th, 2020, Licensor may (i) terminate this Agreement in its entirety with immediate effect upon delivery to Licensee of a written notice, or (ii) modify the license under Section 2.1, in removing the exclusivity granted by Licensor to Licensee under the Licensed Patents in the Field with respect to any or all Licensed Products in any or all countries, with immediate effect upon delivery to Licensee of a written notice.

12.2.3. Action against the Licensed Patents

By giving notification to Licensee, Licensor may terminate immediately the Agreement without any further financial or other obligations to Licensee, in the event that Licensee, its Affiliates or its sublicensees brings an action against Licensor or against the Licensed Patents, in any country of the Territory, in each case, challenging the validity, enforceability or scope of any Licensed Patent, in particular if Licensee, its Affiliates or its sublicensees, directly or indirectly, files an opposition to the Licensed Patents before any patent office in the Territory.

12.2.4. Commercialization of a Licensed Product outside the Field

By giving written notification to Licensee, Licensor may terminate immediately the Agreement without any further financial or other obligations to Licensee, in the event that Licensee, on its own or on its behalf, Commercializes a Licensed Product outside the Field.

12.2.5. Insolvency / Bankruptcy

Either Party may terminate the Agreement in the event that the other Party becomes insolvent, files a petition in bankruptcy, has such a petition filed against it, determines to file a petition in bankruptcy, or receives notice of a Third Party's intention to file an involuntary petition in bankruptcy, and such proceeding or action remains un-dismissed or un-stayed for a period of sixty (60) days, to the extent permitted by applicable law.

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Each Party shall inform the other Party within thirty (30) days of occurrence of any such event.

12.3. <u>Consequences of Termination</u>

12.3.1. Accrued Obligations

Expiration or termination of this Agreement for any reason shall not release any Party of any obligation or liability which, at the time of such expiration or termination, has already accrued or which is attributable to a period prior to such expiration or termination.

12.3.2. Consequence in Case of Termination of the Agreement in its Entirety

In the event of termination of this Agreement in its entirety by Licensor under Section 12.2, all license rights granted to Licensee pursuant to Article 2 shall terminate and any Development and Commercialization of the Licensed Product by Licensee should cease in all the Territory.

12.3.3. Within ninety (90) days of expiration or termination of this Agreement under this Article 12, a final report shall be submitted by Licensee to Licensor. Any royalty payments, including those incurred but not yet paid, and those related to patent expense, due to Licensor shall become immediately due and payable upon termination or expiration. Unless otherwise specifically provided for under this Agreement, upon termination of this Agreement, Licensee shall provide Licensor with written certification of the destruction of the Licensed Products (i.e. destruction of the products integrating patented vectors as detailed in Licensed Patents).

12.4. Non-Exclusive Remedy

Termination of this Agreement by a Party shall be without prejudice to other remedies such Party may have at law or equity.

12.5. <u>Survival</u>

Notwithstanding the expiry of the term or prior termination of this Agreement the provisions of Articles 1 (as applicable), 6 (Intellectual Property), 7 (Confidentiality), 8 (Record Keeping - Audit), 14 (Indemnification, Insurance and Liability), 15 (Publication and Publicity), 16 (Dispute Resolution) and 17 (Miscellaneous) and Sections 5.3.3, 5.6 (Payment Method), 5.7 (Late Payment), 12.3 (Consequences of Termination), 12.4 (Non-Exclusive Remedy) and 12.5 (Survival), and any other provisions that by their nature or intent are intended to survive post expiration or termination, shall remain in full force and effect.

ARTICLE 13. <u>REPRESENTATION AND WARRANTIES</u>

13.1. Licensor represents and warrants to Licensee, as of the Effective Date, that: (a) the information on the Licensed Patents listed in Exhibit A is accurate; (b) the Licensed Patents constitute all of the Patents owned or controlled by Licensor that are related to the construction of a self-complementary AAV9-SMN and AAV9-SMN systemic administration; (c) Licensor has the right to grant to Licensee the rights and licenses in and to the Licensed Patents set forth in this Agreement; (d) Licensor has not received any notice of infringement of any Licensed Patents other those described in Exhibit A at the Effective Date, and to Licensor's knowledge, no third party is infringing, or challenging the inventorship, ownership or enforceability of, any Licensed Patents in the Field; and (e) Licensor has not entered, and

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shall not enter, into any agreement with any Third Party (including any related party agreements) that is in conflict with the rights granted to Licensee under this Agreement.

13.2. Except as expressly set forth in Section 13.1 of this Agreement, Licensor does not warrant the validity of the Licensed Patents and makes no representations whatsoever with regard to the scope of the Licensed Patents, or that the Licensed Patents may be exploited without infringing other patents or other intellectual property rights of Third Parties. Licensee acknowledges that the status of the Licensed Patents are available to the public and that Licensee has been provided with all reasonable information regarding the Licensed Patents available at the Effective Date and that Licensee is full aware of the filing procedures and the status of the Licensed Patents.

13.3. Except as expressly set forth in Section 13.1 of this Agreement, Licensor makes no warranties, expressed or implied, of merchantability or fitness for a particular purpose of any subject matter defined by the claims of the Licensed Patents or tangible materials related thereto.

13.4. Subject to Section 14.1.1, Licensee shall indemnify and hold Licensor, its directors, employees, students, agents, and consultants harmless from and against all liability, demands, damages, expenses, and losses, including but not limited to death, personal injury, illness, or property damage in connection with or arising out of the Development, Manufacture, Commercialization, or use of any Licensed Patents or Licensed Products by Licensee, its Affiliates or sublicensees.

13.5. If data passed on for the purpose of use of one Party (the "**receiving Party**") contain personal data, the other Party guarantees to the receiving Party that he complied with all the obligations imposed under the term of the January 6th, 1978 law "Computing and Liberties" and of the Directive 95/46/EC of the European Parliament and of the Council of 24 October 1995 "on the protection of individuals with regard to the processing of personal data and on the free movement of such data" that will be repealed, as from the May 25, 2018, by the regulation (EU) 2016/679 of the European Parliament and of the Council of 27 April 2016 "on the protection of natural persons with regard to the processing of personal data and on the free movement of such data", and that he informed the concerned physical persons of use which is made of the aforementioned personal data. As such, each Party guarantees the other Party against any appeal, complaint or demand from a person whose personal data would be reproduced and host via Licensee.

ARTICLE 14. INDEMNIFICATION, INSURANCE AND LIABILITY

14.1. <u>Indemnification</u>

14.1.1. Indemnity.

- a. Subject to the Section 14.1.1.b) below, the defaulting Party (the "**Indemnifying Party**") will indemnify, defend, and hold harmless the other Party (the "**Indemnified Party**") and its Affiliates, its sublicensees and its directors, officers, employees, agents (collectively the "**Indemnifees**"), against any Losses incurred by or imposed upon any of the Indemnitees in connection with any Third Party claims, suits, investigations, actions, judgments or demands arising out of or related to :
- i. any breach of this Agreement by the Indemnifying Party, its Indemnitees, Affiliates or sublicensees; or
- ii. the wrongful intentional acts or omissions of the Indemnifying Party, its Indemnitees, Affiliates or sub-licensees, in connection with the performance of its obligations or exercise of its rights under this Agreement;

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b. Except, in each case, to the extent that the respective Losses are caused by the negligence or willful misconduct of, or breach of this Agreement or violation of law by, the Indemnified Party, Indemnitees, its Affiliates, sublicensees.

14.1.2. <u>Settlement</u>. Notwithstanding anything to the contrary in this Agreement, neither Party will enter into any settlement, consent judgment, or other voluntary final disposition of any claim that has an adverse effect on the rights of the other Party, or admits any wrongdoing or fault by the other Party, or imposes on the other Party any payment or other liability, without the prior written consent of such other Party, except when Licensor defends the Licensed Patents in application of Article 11.

14.2. Insurance. The Licensee agrees to maintain a liability insurance program consistent with sound business practice.

14.3. <u>Liability.</u> Notwithstanding anything in this Agreement or otherwise, neither Party, their directors, employees, students, agents, and consultants as applicable, will be liable to the other with respect to any subject matter of this Agreement for any indirect, punitive, special or consequential damages, including incidental, or lost profits, even if such Party has been informed, should have known or in fact knew of the possibility of such damages.

ARTICLE 15. <u>PUBLICATION AND PUBLICITY</u>

15.1. <u>Scientific Publication</u>

Licensee and its Affiliates shall have the right to publish or publicly disclose, as part of scientific publications or scientific presentations, the results generated in the course of performing by Licensee any research related to the Licensed Products. Licensor may publish or publicly disclose any information or results generated in the course of performing any research related to the Licensed Patents without the prior written consent of Licensee.

15.2. Press Release and Public Communication

15.2.1 The Parties shall issue the initial press release set forth on Exhibit B hereto following the Effective Date.

15.2.2 Except as required by law, neither Licensor nor Licensee (or its Affiliates or sublicensees) shall issue or cause the publication of any other press release or public announcement (orally or in writing) regarding the existence or terms of this Agreement without the express prior written approval of the other Party, which approval shall not be unreasonably withheld, conditioned or delayed. The Licensor or the Licensee (or its Affiliates or sublicensees) shall reasonably consider, timely comments from the other Party on such publication or press release.

15.2.3 Unless prohibited by law and unless otherwise requested by Licensor, Licensee shall include the words "Developed in partnership with Genethon" in any press release, public presentation, or medical communication related to the Licensed Product where Licensee believes it is appropriate and reasonable to do so and in all cases where Licensee includes the names of its other licensing partners. In addition, Licensee would include the words "Developed in partnership with Genethon" in significant global press releases announcing initiation of a Clinical Trial, results of a Clinical Trial, submission for regulatory approval and the receipt of regulatory approvals. In France, Licensee would help promote Licensor's

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branding by including the wording when announcing any joint advocacy communication, local advisory board and local conference/presentation.

15.2.4 The Parties acknowledge that the French Association against Myopathies (AFM-Téléthon, Association Française contre les Myopathies), which is Genethon's founder and principal funder, in view of accomplishing its recognized role of working in the public interest, that is, by curing rare diseases and reducing the disabilities to which they give rise, has an obligation to provide the general public with information on the research programs and work to which it provides a financial contribution, in order to facilitate, directly or indirectly, an understanding of these diseases, the development of treatments, and the prevention of disabilities.

Subject to Licensee's prior approval which should not be unreasonably withheld, each Party agrees that AFM-Telethon may use its name and report thereon, orally and/or in writing, to the general public, notably during fundraising campaigns and during the Telethon and annual general meeting, without releasing details which may be detrimental to such Party's intellectual property rights or Confidential Information.

ARTICLE 16. DISPUTE RESOLUTION

16.1. In the event of any dispute arising out of or relating to this Agreement, the Parties will use all reasonable efforts to arrive at a mutually acceptable resolution.

16.2. If a dispute is not resolved within sixty (60) days from the date that the other Party receives notice of the dispute, the matter will be referred to the CEOs of both Parties (or their designees) for resolution within thirty (30) days after such escalation to such officers.

16.3. If an agreement is not reached within such thirty (30) day period, then the dispute will be resolved consistent with Section 17.2.

16.4. The provisions of this Article 16 shall survive for five (5) years from the date of termination or expiration of this Agreement.

16.5. Subject to the non-terminating Party's right to seek an injunction or protective order, the provisions of this Article 16 shall not affect the right of either Party to terminate this Agreement pursuant to Section 12.2 of this Agreement.

ARTICLE 17. MISCELLANEOUS

17.1. <u>Governing Law</u>

This Agreement and all disputes arising out of or related to this Agreement, or the performance, enforcement, breach or termination hereof, and any remedies relating thereto, will be construed, governed, interpreted and applied in accordance with the laws of Belgium, without regard to conflict of laws principles, except that questions affecting the construction and effect of any patent will be determined by the law of the country in which the patent will have been granted.

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17.2. Attribution of Jurisdiction

Any dispute arising out of or relating to this Agreement, any terms of this Agreement or any breach of this Agreement (in particular with regard to the negotiation, performance or termination of the Agreement) that is not resolved pursuant to Article 16 will be resolved by the courts of Brussels (Belgium) which will have exclusive jurisdiction, without regard to conflict of jurisdiction principles thereof. The Parties hereby expressly exclude the application of the United National Convention on Contracts for the International Sales of Goods.

17.3. Assignment of Rights and Obligations

This Agreement and its rights or obligations may not be assigned or otherwise transferred by Licensee without the prior written consent of Licensor; provided that, Licensee may make such an assignment or transfer without Licensor's consent, but with notice promptly following such assignment or transfer, to its Affiliates or to a Third Party successor to all or substantially all of the business of Licensee to which this Agreement relates, whether in a merger, sale of stock, sale of assets or other transaction.

17.4. <u>Further Actions</u>

Each Party agrees to execute, acknowledge and deliver such further instruments, and to do all such other acts, as may be necessary or appropriate in order to carry out the purposes and intent of the Agreement.

17.5. <u>Force Majeure</u>

Except with respect to payment of money, no Party shall be liable to the other Party for failure or delay in the performance of any of its obligations under this Agreement for the time and to the extent such failure or delay is caused by earthquake, riot, civil commotion, war, terrorist acts, strike, flood, or governmental acts or restriction, or other cause that is beyond the reasonable control of the respective Party (**'Force Majeure'**). The Party affected by such Force Majeure will provide the other Party with full particulars thereof as soon as it becomes aware of the same (including its best estimate of the likely extent and duration of the interference with its activities), and will use reasonable efforts to overcome the difficulties created thereby and to resume performance of its obligations as soon as practicable. If the performance of any such obligation under this Agreement is delayed owing to an event of Force Majeure for any continuous period of more than ninety (90) days, the Parties will consult with respect to an equitable solution, including the possibility of the termination of this Agreement in accordance with Section 12.2.

17.6. <u>Representation by Legal Counsel</u>

Each Party hereto represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption shall exist or be implied against the Party which drafted such terms and provisions.

17.7. <u>Notices</u>

Any notice, request, delivery, approval or consent required or permitted to be given under this Agreement shall be in writing and shall be deemed to have been sufficiently given if delivered in person, transmitted by facsimile (receipt verified) or by express courier service (signature required) or five (5) days after it was sent by registered letter, return receipt requested (or its equivalent), provided that no postal strike or

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other disruption is then in effect or comes into effect within two (2) days after such mailing, to the Party to which it is directed at its address or facsimile number shown below or such other address or facsimile number as such Party will have last given by notice to the other Party.

If to Licensor: 1bis, Rue de l'Internationale - 91000 EVRY, FRANCE Attention: Alexandre LEMOALLE Tel: +33 1 69 47 25 87 Mail: alemoalle@genethon.fr If to Licensee: 2275 Half Day Road, Suite 200 Bannockburn, IL 60015 Attention: General Counsel Fax: 847-510-0775 Mail: mjohannesen521@avexis.com

17.8. Entire Agreement

This Agreement, together with the exhibits A to B attached hereto, set forth the entire agreement and understanding of the Parties hereto as to the subject matter hereof, and supersedes all prior and contemporaneous discussions, agreements and writings in respect.

17.9. Amendment

No amendment, modification or supplement of any provision of this Agreement shall be valid or effective unless made in writing and signed by a duly authorized officer of each Party.

17.10. <u>Waiver</u>

No provision of the Agreement shall be waived by any act, omission or knowledge of a Party or its agents or employees except by an instrument in writing expressly waiving such provision and signed by a duly authorized officer of the waiving Party. The waiver by any of the Parties of any breach of any provision hereof by another Party shall not be construed to be a waiver of any succeeding breach of such provision or a waiver of the provision itself.

17.11. <u>Severability</u>

If any clause or portion thereof in this Agreement is for any reason held to be invalid, illegal or unenforceable, the same shall not affect any other portion of this Agreement, as it is the intent of the Parties that this Agreement shall be construed in such fashion as to maintain its existence, validity and enforceability to the greatest extent possible. In any such event, this Agreement shall be construed as if such clause of portion thereof had never been contained in this Agreement, and there shall be deemed substituted therefor such provision as will most nearly carry out the intent of the Parties as expressed in this Agreement to the fullest extent permitted by Law.

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17.12. <u>Relationship of the Parties</u>

Nothing herein will be deemed to establish a relationship of principal and agent between Licensor and Licensee, nor any of their agents or employees, nor will this Agreement be construed as creating any form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party. Nothing in this Agreement, express or implied, is intended to confer on any person other than the Parties or their permitted assigns any benefits, rights or remedies.

17.13. Third Party Beneficiaries

All rights, benefits and remedies under this Agreement are solely intended for the benefit of the Parties (including any successor in interest or permitted assigns), and no Third Party shall have any rights whatsoever to (a) enforce any obligation contained in this Agreement, (b) seek a benefit or remedy for any breach of this Agreement, or (c) take any other action relating to this Agreement under any legal theory, including actions in contract, tort (including negligence, gross negligence and strict liability), or as a defense, setoff or counterclaim to any action or claim brought or made by the Parties.

17.14. <u>Counterparts</u>

This Agreement may be executed in any number of counterparts, each of which need not contain the signature of more than one Party but all such counterparts taken together shall constitute one and the same agreement.

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Executed in two originals, one for each Party

For AVEXISFor GENETHON at Bannockburn, ILat Evry the 9_{th} of March, 2018the 9_{th} of March, 2018

/s/ RA Session II/s/ Frederic REVAH

Name: RA Session II Name : Frederic REVAH Title: Senior Vice President Corporate Title : Chief Executive Officer Strategy and Business Development

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Licensed Patents at the Effective Date

Countr y	Filing date	Administration	Product*	Filing number	Publication number
"CNS ge	ene deliver	y using peripheral administration o	f AAV vectors" GENETH	ON [B00701 MB], CNR	S [DI 02300-01]
Europe	23/07/20 07	N/A (withdrawn)	N/A (withdrawn)	EP 07301263.5	EP 2019143
РСТ	22/07/20 08	N/A (expired)	N/A (expired)	PCT/EP2008/059595	WO 2009/013290
Europe	22/07/20 08	All	scAAV9-SMN	EP 08786331.2	EP2185712
Europe	22/07/20 08	N/A (other serotype)**	N/A (other serotype**	EP 12 172 848.9	EP2514827
USA	22/07/20 08	Intravascular or Intraperitoneal	scAAV9-SMN	12/452,789	US 2010/0130594
Canada	22/07/20 08	Intravascular, intraperitoneal, intramuscular	scAAV9-SMN	CA20082694241	CA 2694241
CNRS [DI 02300- 05/10/20 07	e Delivery to Motor Neurons Using 02] N/A (withdrawn)	Peripheral Injection of AANN N/A (withdrawn)	AV Vectors" GENETHO EP 07301435.9	N [B00703 MB], EP2058401
PCT		N/A (expired)	N/A (withardwir) N/A (expired)	PCT/EP2008/063297	W02009/043936
101	08			1 01/11 2000/00025/	
Canada	03/10/20 08	Intravascular, intraperitoneal, intramuscular	scAAV9-SMN	CA20082701561	CA 2701561
China	03/10/20 08	N/A (rejected)	N/A (rejected)	CN20088117413	CN 101883859
China	03/10/20 08	Peripheral	scAAV9-SMN	CN201510438197.1	CN 105087650
Europe	03/10/20 08	Intravenous, Intraperitoneal or intramuscular	scAAV9-SMN	EP 08836776.8	EP 2212424
Europe	03/10/20 08	Peripheral	ssAAV9-SMN	EP 12 180 951.1	EP 2527457
USA	03/10/20 08	Intravenous or intra-arterial	scAAV9-SMN	12/734,016	US 2010/0240739
USA	22/09/20 17	All	scAAV9-SMN	15/713,347	
Japan	03/10/20 08	Peripheral	scAAV9-SMN	JP20100527467T	JP 2010540598
Japan	01/07/20 14	Peripheral	ssAAV9-SMN	2014-136031	JP2014221789

*: By product we mean the Licensed Product constructions covered by the Licensed Patents in the Field of the license granted under section 2.1, i.e. restricted to AAV9.

: The European divisional with the publication number **EP2514827 covers a serotype that is not included in the Field of the License.

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AveXis Enters into Licensing Agreement with Genethon

Includes exclusive worldwide rights to AAV9-SMN product and route of administration

Chicago, Ill. and Evry, France (March XX, 2018) – AveXis, Inc. (NASDAQ:AVXS) and Genethon today announced they have entered into an exclusive, worldwide license agreement for *in vivo* gene therapy delivery of AAV9 vector into the central nervous system (CNS) for the treatment of spinal muscular atrophy (SMA).

"Adding to our robust intellectual property estate, this agreement further strengthens our position by providing freedom to operate when using intravenous or intrathecal routes of administration to deliver the AAV9 vector into the CNS for the treatment of SMA," said Sean Nolan, President and Chief Executive Officer of AveXis. "With our proprietary gene therapy, AVXS-101, currently being evaluated in patients with SMA in ongoing clinical trials in the U.S., and soon in Europe, we are pleased to have this exclusive worldwide agreement in place."

Under the terms of the agreement, Genethon granted AveXis a license to patents in the U.S., Europe and Japan, for the AAV9 SMN product and *in vivo* gene therapy delivery of AAV9 vector into the CNS using intrathecal or intravenous routes of administration for the treatment of SMA.

"Genethon is pleased to enter into this agreement with AveXis and to contribute to the efforts for the development of treatments for SMA patients who have urgent medical needs," said Frédéric Revah, Chief Executive Officer of Genethon. "It demonstrates Genethon's capability

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to develop effective first-in-class technologies and the excellence of our translational research driven by the commitment to treat patients living with rare diseases."

About SMA

SMA is a severe neuromuscular disease characterized by the loss of motor neurons leading to progressive muscle weakness and paralysis. SMA is caused by a genetic defect in the *SMN1* gene that codes SMN, a protein necessary for survival of motor neurons. The incidence of SMA is approximately one in 10,000 live births and is the leading genetic cause of infant mortality.

The most severe form of SMA is Type 1, a lethal genetic disorder characterized by motor neuron loss and associated muscle deterioration, which results in mortality or the need for permanent ventilation support before the age of two for greater than 90 percent of patients. SMA Type 2 typically presents between six and 18 months of age, and those affected will never walk without support and most will never stand without support. SMA Type 2 results in mortality in more than 30 percent of patients by the age of 25.

About AVXS-101

AveXis' initial product candidate, AVXS-101, is its proprietary gene therapy currently in development for the one-time treatment of SMA Types 1 and 2, designed to address the monogenic root cause of SMA and prevent further muscle degeneration by addressing the defective and/or loss of the primary SMN gene. AVXS-101 also targets motor neurons, providing rapid onset of effect and crossing the blood brain barrier to allow effective targeting of both central and systemic features.

About AveXis, Inc.

AveXis, Inc. is a clinical-stage gene therapy company, dedicated to developing and commercializing novel treatments for patients suffering from rare and life-threatening neurological genetic diseases. Our initial product candidate, AVXS-101, is our proprietary gene therapy currently in development for the treatment of spinal muscular atrophy, or SMA, Type 1, the leading genetic cause of infant mortality, and SMA Type 2. The U.S. Food and Drug Administration, or FDA, has granted AVXS-101 Orphan Drug Designation for the treatment of all types of SMA and Breakthrough Therapy Designation, as well as Fast Track Designation for the treatment of SMA Type 1. In addition to developing AVXS-101 to treat SMA Type 1 and Type 2, we also plan to develop other novel treatments for rare neurological diseases, including Rett syndrome and a genetic form of amyotrophic lateral sclerosis caused by mutations in the superoxide dismutase 1 (*SOD1*) gene.

About Genethon

Created by the AFM-Telethon, the French Muscular Dystrophy Association (AFM), Genethon, located in Evry, France, is a nonprofit R&D organization dedicated to the development of biotherapies for orphan genetic diseases, from the research to clinical validation. Genethon is specialized in the discovery and development of gene therapy drugs and has multiple ongoing programs at clinical, preclinical and research stage for neuromuscular, blood, immune system, and liver diseases.

AveXis Forward-Looking Statements

This press release contains "forward-looking statements," within the meaning of the Private Securities Litigation Reform Act of 1995, regarding, among other things, AveXis' freedom to operate afforded by the license agreement with Genethon and AveXis' research, development and regulatory plans for AVXS-101. Such forward-looking statements are based on current

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expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual results to differ materially from those projected in its forward-looking statements. Meaningful factors which could cause actual results to differ include, but are not limited to, the scope, progress, expansion, and costs of developing and commercializing AveXis' product candidates; regulatory developments in the U.S. and EU, as well as other factors discussed in the "Risk Factors" and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of AveXis' Annual Report on Form 10-K for the year ended December 31, 2017, filed with the SEC on February 28, 2018. In addition to the risks described above and in the Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and other filings with the SEC, other unknown or unpredictable factors also could affect AveXis' results. There can be no assurance that the actual results or developments anticipated by AveXis will be realized or, even if substantially realized, that they will have the expected consequences to, or effects on, AveXis. Therefore, no assurance can be given that the outcomes stated in such forward-looking statements and estimates will be achieved.

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